Harvard Pilgrim Health Care | TUFTS Health Plan

Medical Necessity Guideline
Medical Benefit Drug
Xenpozyme™ (olipudase alfa-rpcp)

Effective: January 1, 2026

Guideline Type	☒ Prior Authorization ☐ Non-Formulary ☐ Step-Therapy ☐ Administrative

Public Plans Products

☒ Tufts Health One Care* – A Medicare-Medicaid Plan (a dual-eligible product); Fax 617-673-0956

*The MNG applies to Tufts Health One Care members unless a less restrictive LCD or NCD exists.

Senior Products

☒ Tufts Health Plan Senior Care Options (SCO), (a dual-eligible product); Fax 617-673-0956

☒ Tufts Medicare Preferred HMO, (a Medicare Advantage product); Fax 617-673-0956

☒ Tufts Medicare Preferred PPO, (a Medicare Advantage product); Fax 617-673-0956

Note: While you may not be the provider responsible for obtaining prior authorization, as a condition of payment you will need to ensure that prior authorization has been obtained.

Overview

Acid sphingomyelinase deficiency (ASMD) is a rare, progressive genetic disorder that results from a deficiency of the enzyme acid sphingomyelinase (ASM). ASMD is a lysosomal storage disease that is caused by mutations in the sphingomyelin phophodiesterase-1 (SMPD1) gene. ASMD is considered a disease spectrum that is divided into three types: Type A (most severe), Type B (milder form), and Type A/B. An ASMD diagnosis is suspected in patients who present with characteristic symptoms but must be confirmed through acid sphingomyelinase enzyme activity and genetic testing of the SMPD1 gene.

Xenpozyme is an enzyme replacement therapy intended to reduce sphingomyelin accumulation in the liver, spleen, and lung.

Approval was based on positive data from the ASCEND and ASCEND-Peds trials, which demonstrated that Xenpozyme improved lung function and platelet count, and reduced spleen and liver volumes.

Food and Drug Administration - Approved Indications

Xenpozyme (olipudase alfa-rpcp) is a hydrolytic lysosomal sphingomyelin-specific enzyme indicated for treatment of non–central nervous system manifestations of acid sphingomyelinase deficiency (ASMD) [types B and A/B] in adult and pediatric patients.

Clinical Guideline Coverage Criteria

The plan may authorize Xenpozyme for Members when **ALL** the following clinical criteria is met:

Initial Authorization Criteria

1. Documented diagnosis of acid sphingomyelinase deficiency (ASMD) confirmed by **both** of the following:
    a. The diagnosis of ASMD has been established by acid sphingomyelinase (ASM) enzymatic assay testing
    b. The diagnosis of ASMD has been confirmed by mutation testing

AND

2. Documentation the patient has **one (1)** of the following:
    a. ASMD Type B
    b. ASMD Type A/B

AND

3. Documentation the patient has **two (2) or more** non-central nervous system signs of ASMD type B or type A/B according to the prescriber (examples: hepatosplenomegaly, interstitial lung disease, decreased diffusing capacity of the lungs, progressive liver disease with cirrhosis or fibrosis, dyslipidemia, osteopenia, thrombocytopenia, anemia, leukopenia)

AND

4. Prescribed by or in consultation with a specialist familiar with the treatment of lysosomal storage disorders

Renewal Authorization Criteria

1. Documented diagnosis of acid sphingomyelinase deficiency (ASMD) and judged by the provider to be ASMD type B or type A/B.

AND

2. Xenpozyme is being prescribed by or in consultation with a specialist familiar with the treatment of lysosomal storage disorders.

AND

3. Documentation from the treating provider that confirms the Member is experiencing a positive clinical response to Xenpozyme treatment (e.g. improvement in diffusion capacity of the lungs for carbon monoxide, reduction in spleen volume, reduction of symptoms/manifestations of acid sphingomyelinase deficiency, reduction in level of supportive care).

Limitations

- Xenpozyme will be authorized in 12-month intervals.
- Members new to the plan stable on Xenpozyme should be reviewed against Reauthorization Criteria.

Codes

The following code(s) require prior authorization:

Table 1: HCPCS Codes

HCPCS Codes	Description
J0218	Injection, olipudase alfa-rpcp, 1 mg

References

1. Xenpozyme (olipudase alfa-rpcp). [Prescribing Information]. Genzyme Corporation; Cambridge, MA. December 2023.
2. McGovern MM, et al. Consensus recommendation for a diagnostic guideline for acid sphingomyelinase deficiency. Genet Med. 2017 Sep; 19(9):967-74.
3. Diaz GA, et al. One-year results of a clinical trial of olipudase alfa enzyme replacement therapy in pediatric patients with acid sphingomyelinase deficiency. Genet Med. 2021;23(8):1543-1550.
4. McGovern MM, et al. Disease manifestations and burden of illness in patients with acid sphingomyelinase deficiency (ASMD). Orphanet J Rare Dis. 2017;12(1):41.
5. Thurberg BL, et al. Long-term efficacy of olipudase alfa in adults with acid sphingomyelinase deficiency (ASMD): further clearance of hepatic sphingomyelin is associated with additional improvements in pro- and anti-atherogenic lipid profiles after 42 months of treatment. Mol Genet Metab. 2020;131(1-2):245-252.
6. Wasserstein MP, et al. Olipudase alfa for treatment of acid sphingomyelinase deficiency (ASMD): safety and efficacy in adults treated for 30 months. J Inherit Metab Dis. 2018;41(5):829-838.
7. Wasserstein M, et al. A randomized, placebo-controlled clinical trial evaluating olipudase alfa enzyme replacement therapy for chronic acid sphingomyelinase deficiency (ASMD) in adults: one-year results. Genet Med. 2022;24(7):1425-1436.
8. Wasserstein MP, Jones SA, Soran H, et al. Successful within-patient dose escalation of olipudase alfa in sphingomyelinase deficiency. Mol Genet Metab. 2015;116(1-2):88-97.

Approval And Revision History

December 13, 2022: Reviewed and approved by Pharmacy and Therapeutics Committee (P&T)

December 21, 2022: Reviewed by the Medical Policy Approval Committee (MPAC)

Subsequent endorsement date(s) and changes made:

- Administrative update: March 2023 added Medical Benefit Drugs to title and updated MATogether and RITogether fax numbers to 617-673-0939
- Coding update per HCPCS level II quarterly release. Effective date April 1, 2023, the following HCPCS codes have been added: J0218
- November 14, 2023: Removed the Limitation The Plan will not cover Xenpozyme (olipudase alfa-rpcp) for Members with a diagnosis of ASMD type A or any other non-FDA-approved indication(s). Updated Initial Authorization Criteria to confirmation of ASMD by, confirmation the patient has ASMD type B or A/B, presence of two or more non-central nervous system signs of ASMD type B or type A/B, and provider specialty requirements. Removed baseline ALT and AST, diffusion capacity of the lungs for carbon monoxide and spleen volume requirements. Removed requirement for provider attestation the member does not have one or more of the following circumstances: The Member has acute or rapidly progressive neurologic abnormalities, The Member requires use of invasive ventilatory support or requires noninvasive ventilatory support while awake and for greater than 12 hours a day, and The Member has a platelet count less than 60 x 103/µL. Updated Reauthorization Criteria to remove diagnosis requires confirmation by enzyme assay (effective 2/1/2024).
- November 2023: Administrative Update in support of calendar year 2024 Medicare Advantage and PDP Final Rule.
- October 2024: Administrative Updates: Rebranded Tufts Health Unify to Tufts Health One Care
- October 8, 2024: No changes.
- December 2024: Joint Medical Policy and Health Care Services UM Committee review (eff 1/1/25)

- July 2025: Administrative update to remove Harvard Pilgrim Health Care Stride Medicare Advantage from the Medical Necessity Guideline template.
- December 9, 2025: No changes (eff 1/1/26)
- December 2025: Joint Medical Policy and Health Care Services UM Committee review (effective 1/1/26)

Background, Product and Disclaimer Information

Point32Health prior authorization criteria to be applied to Medicare Advantage plan members is based on guidance from Medicare laws, National Coverage Determinations (NCDs) or Local Coverage Determinations (LCDs). When no guidance is provided, Point32Health uses clinical practice guidance published by relevant medical societies, relevant medical literature, Food and Drug Administration (FDA)-approved package labeling, and drug compendia to develop prior authorization criteria to apply to Medicare Advantage plan members. Medications that require prior authorization generally meet one or more of the following criteria: Drug product has the potential to be used for cosmetic purposes; drug product is not considered as first-line treatment by medically accepted practice guidelines, evidence to support the safety and efficacy of a drug product is poor, or drug product has the potential to be used for indications outside of the indications approved by the FDA. Prior authorization and use of the coverage criteria within this Medical Necessity Guideline will ensure drug therapy is medically necessary, clinically appropriate, and aligns with evidence-based guidelines. We revise and update Medical Necessity Guidelines annually, or more frequently if new evidence becomes available that suggests revisions.

Treating providers are solely responsible for the medical advice and treatment of Members. The use of this guidelines not a guarantee of payment or a final prediction of how specific claim(s) will be adjudicated. Claims payment is subject to eligibility and benefits on the date of service, coordination of benefits, referral/authorization, utilization management guidelines when applicable, and adherence to plan policies, plan procedures, and claims editing logic.